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Ribozyme Pharmaceuticals Receives Approval For First Foray Into Clinic

By Lisa Seachrist

Washington Editor

Ribozyme Pharmaceuticals Inc., in collaboration with Chiron Corp. has received approval to start a clinical trial of ribozymes in HIV-infected patients.

The Phase VIIa gene therapy trial will test the safety of inserting ribozymes, RNA enzymes that cleave RNA, targeted against HIV into the stem cells of HIV-infected patients with an eye to creating immune cells that are resistant to infection by HIV. This is the first investigational new drug application that Ribozyme of Boulder, Colo., submitted for a product based on its RNA enzyme technology.

"This represents quite a major change for the company," said Ralph Christofferson president and CEO of Ribozyme. "When we started this company in 1992, we had a wonderful, Nobel-prize winning idea but no technology base to support our development. Now, we have evolved from a technology company to one that is able to develop products."

While Ribozyme is hitting a milestone by making its first foray into the clinic, the trial will include only five HIV-infected patients who have yet to progress to AIDS. The trial, which is set to begin during the first half 1997, will take place at the City of Hope National Medical Center in Los Angeles as a collaboration between the two companies and Childrens Hospital also in Los Angeles.

After isolating CD 34 cells — thought by many to be stem cells — from the patients' blood, the researchers will infect the cells with a retrovirus containing genes for two different ribozymes. The retrovirus was designed by Chiron, of Emeryville, Calif. The 15 nucleotide-long ribozymes, which are targeted to the *lat* region and the *tat/rev* junction of the HIV virus, were created by Ribozyme.

The companies hope that the cells infected with the retrovirus will produce ribozymes that can prevent infection with HIV by chopping up the HIV RNA. These resistant cells will then be injected back into the patients' blood where it is hoped they will enter the bone marrow and become permanent progenitors of HIV-resistant immune cells.

"What we hope to do is to reconstitute elements of the immune system with these resistant stem cells," Christofferson said.

Cell culture data supports the feasibility of their plan. When CD 34 cells that were growing in culture were infected with the ribozyme-containing retrovirus, the cells were protected for their lifetime against infection with HIV.

However, Christofferson is quick to point out that there are many variables in the experiment. First, no one knows if the ribozymes will act in the body. In addition, while CD 34 cells are clearly early cells produced in the bone marrow, no one knows for sure that they are stem cells. And, the researchers don't even know if the CD 34 cells will actually reengraft in the bone marrow.

"This is only the beginning of our efforts at creating an HIV therapy," Christofferson said. "But, it is a major step to have the data to go forward into the clinic."

Because the companies have no way of knowing if the CD 34 cells will reengraft, they can't predict how long it will take for this first trial to be completed.

Scott Sacane, senior biotech analyst with Montgomery Securities, in New York, said that "it is a very big step for the company." He noted that the company will have to "switch mindset from a company of research scientists developing research products to a company that must plan for the marketing and launch of a product."

While Sacane noted that Ribozyme's stock (NASDAQ:RZYM) gained about \$2 on the news last week,

investors are really going to take a look at
trials. The company has an tremendously
essentially halt protein expression, they have po

he said. "Because ribozymes
of diseases."

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